

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization
International Bureau



(43) International Publication Date
12 May 2005 (12.05.2005)

PCT

(10) International Publication Number
WO 2005/041857 A3

(51) International Patent Classification:
C07H 21/04 (2006.01)

(21) International Application Number:
PCT/IL2004/000924

(22) International Filing Date: 6 October 2004 (06.10.2004)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/509,279 7 October 2003 (07.10.2003) US

(71) Applicants (for all designated States except US): **QUARK BIOTECH, INC.** [US/US]; 6536 Kaiser Drive, Fremont, CA 94555 (US). **ASTELLAS PHARMA, INC.** [—/JP]; 3-11 Nihonbashi-Honcho, 2-chome Chuo-ku, 103-8411 Tokyo (JP).

(72) Inventors; and

(75) Inventors/Applicants (for US only): **FEINSTEIN, Elena** [IL/IL]; 12/29 HaHagana Street, Rehovot (IL). **METT, Igor** [IL/IL]; 18/6 Gorodesky Street, Rehovot (IL). **GORODIN, Svetlana** [IL/IL]; 22/25 Abarbanel Street, 75309 Rishon-le-Zion (IL). **SHTUTMAN, Michael** [IL/IL]; 7a Meltzer Street, Rehovot (IL).

(74) Agent: **ABRAHAM, Judith, Dr.**; QBI Entreprises Ltd., Weizmann Science Park, P.O. Box 4071, 70400 Nes Ziona (IL).

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

- with international search report
- before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments

(88) Date of publication of the international search report:
26 July 2007

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: **BONE MORPHOGENETIC PROTEIN (BMP) 2A AND USES THEREOF**

(57) Abstract: The present invention provides compositions and methods for alleviation or reduction of the symptoms and signs associated with damaged neuronal tissues whether resulting from tissue trauma, or from chronic or acute degenerative changes. In particular, some embodiments of the present invention provide one or more pharmaceutical compositions comprising as an active ingredient a BMP2A inhibitor further comprising a pharmaceutically acceptable diluent or carrier. An additional embodiment provides a method for reducing damage to the central nervous system in a patient who has suffered an injury to the central nervous system, comprising administering to the patient a pharmaceutical composition in a dosage sufficient to reduce the damage. Yet another embodiment provides the use of a BMP2A inhibitor for the preparation of a medicament for promoting or enhancing recovery in a patient who has suffered an injury to the central nervous system. Preferable inhibitors according to some embodiments of the invention are siRNA molecules and neutralizing antibodies. An additional embodiment provides a method for identifying a chemical compound that modulates apoptosis. Further, a process for diagnosing a neurodegenerative disease or an ischemic event in a subject is provided. The preferred methods, materials, and examples that will now be described are illustrative only and are not intended to be limiting; materials and methods similar or equivalent to those described herein can be used in practice or testing of the invention. Other features and advantages of the invention will be apparent from the following detailed description, and from the claims.

WO 2005/041857 A3